Reference number(s)
3463-A

SPECIALTY GUIDELINE MANAGEMENT

VYONDYS 53 (golodirsen)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Vyondys 53 is indicated for the treatment of Duchenne muscular dystrophy (DMD) in patients who have a confirmed mutation of the DMD gene that is amenable to exon 53 skipping.

This indication is approved under accelerated approval based on an increase in dystrophin production in skeletal muscle observed in patients treated with Vyondys 53. Continued approval for this indication may be contingent upon verification of a clinical benefit in confirmatory trials.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
Laboratory confirmation of Duchenne muscular dystrophy (DMD) diagnosis with a *DMD* gene mutation that is amenable to exon 53 skipping (refer to examples in Appendix).

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of DMD.

IV. CRITERIA FOR INITIAL APPROVAL

A. Duchenne Muscular Dystrophy
Authorization of 6 months may be granted for treatment of DMD when all of the following criteria are met:
1. Genetic testing was conducted to confirm the diagnosis of DMD and to identify the specific type of *DMD* gene mutation.
2. The *DMD* gene mutation is amenable to exon 53 skipping (refer to examples in appendix).
3. Treatment with Vyondys 53 is initiated before the age of 16.
4. Member is able to achieve an average distance of at least 250 meters while walking independently over 6 minutes.

V. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for members requesting continuation of therapy when the member has demonstrated a response to therapy as evidenced by remaining ambulatory (e.g. able to walk with or without assistance, not wheelchair dependent).

VI. APPENDIX

Examples of DMD gene mutations (exon deletions) amenable to exon 53 skipping

1. Deletion of exon 52
2. Deletion of exon 45-52
3. Deletion of exon 47-52
4. Deletion of exon 48-52
5. Deletion of exon 49-52
6. Deletion of exon 50-52

VII. REFERENCES

1. Vyondys 53 [package insert]. Cambridge, MA: Sareta Therapeutics; December 2019.
2. Watanabe N, Nagata T, Satou Y, et al. NS-065/NCNP-01: An Antisense Oligonucleotide for Potential Treatment of Exon 53 Skipping in Duchenne Muscular Dystrophy. *Mol Ther Nucleic Acids*. 2018;13:442–449. doi:10.1016/j.omtn.2018.09.017
3. Vyondys 53™ (golodirsen) eDossier. AMCP Formulary Decisions. AmerisourceBergen Corporation. Conshohocken, PA. Available at: www.formularydecisions.com. Accessed December 20, 2019.